

**OBJECTIVES:** To assess the effects of early psychosocial intervention on delaying the institutionalization of patients with mild Alzheimer's disease (AD) and on caregivers' health-related quality of life (HRQL). **METHODS:** Totally, 240 patient-caregiver dyads were recruited to a pragmatic, controlled, and randomized (1:2) clinical trial in 3 hospital districts in Finland between 2002–2006. A primary outcome measure was the intervention's impact on cumulative risk of institutionalization over 36 months of follow up. In the analysis of the primary outcome, Fine & Gray's (1999) proportional subhazards (sHR) model was applied to adjust the impact of competing risk (i.e., death). Main secondary outcome measures were caregivers' global burden (measured by Global Health Questionnaire GHQ) and HRQL measured by the 15D. The secondary outcomes were analyzed using generalized linear mixed models (GLMM) with hierarchical structure to account for repeated measures and possible clustering by the hospital district. **RESULTS:** After 36 months of follow up, cumulative competing risk adjusted incidence of institutionalization rates (95% CI) were 21.2% (12.5% – 29.9%) and 16.1% (10.3% – 21.9%) in the intervention and usual care groups, respectively. Age and sex adjusted sHR was 1.37 (95%CI 0.75 – 2.52). No statistical significant differences in the secondary outcomes were found. **CONCLUSIONS:** The early psychosocial intervention for patients with mild AD and their caregivers did not delay time to nursing home placement. The results of the present study are consistent with a recently published study (Waldorff et al. 2012 BMJ) reporting no effect of semi-tailored intervention for patients with mild AD and their caregivers. Even if the present study did not manage to show differences between the study groups, it provides the valuable longitudinal dataset for studying long-term disease progression (in terms of correlated cognitive, behavioral, and functional disabilities) and its economic and quality of life consequences for patients with AD and their caregivers.

#### PND6

##### MODELING THE IMPACT OF DISEASE MODIFYING TREATMENT ON TIME TO DISABILITY HEALTH STATES IN MULTIPLE SCLEROSIS: AN EVALUATION OF ORAL THERAPIES THROUGH INDIRECT COMPARISONS OF 6-MONTH CONFIRMED DISABILITY PROGRESSION

Bergvall N<sup>1</sup>, Rathi H<sup>2</sup>, Nixon RM<sup>1</sup>, Thom HHZ<sup>3</sup>, Alsop J<sup>4</sup>, Dunsire L<sup>4</sup>

<sup>1</sup>Novartis Pharma AG, Basel, Switzerland, <sup>2</sup>Novartis Healthcare Pvt.Ltd., Hyderabad, India, <sup>3</sup>MRC Biostatistics Unit, Cambridge, UK, <sup>4</sup>Numerus Ltd., Wokingham, UK

**OBJECTIVES:** To estimate the comparative efficacy of oral therapies (fingolimod, dimethyl fumarate [DMF] and teriflunomide) in delaying progression to disability health states in patients with relapsing-remitting multiple sclerosis (RRMS). **METHODS:** Cox proportional hazards regression models were used to analyse 6-month confirmed disability progression (CDP; based on Expanded Disability Status Scale [EDSS] scores) in the pooled fingolimod FREEDOMS trials. Initial models were constructed with eight baseline covariates as main and treatment-interaction effects and final models with the most predictive covariates were selected using a stepwise algorithm. Models predicted hazard ratios (HRs) for 6-month CDP for fingolimod 0.5mg versus placebo for average TEMSO (teriflunomide trial) and pooled DEFINE/CONFIRM (DMF trials) patients. Time from EDSS score 0 to scores of 4 or 6 and to conversion to secondary progressive multiple sclerosis (SPMS) were estimated by fitting a multi-state Markov Transition model to individual patient data from the pooled FREEDOMS placebo groups (HRs accounted for treatment effects) and the London Ontario cohort (SPMS and RRMS-SPMS transitions). **RESULTS:** Without covariate adjustment, the HR for CDP for fingolimod versus placebo in the pooled FREEDOMS trials was numerically lower (i.e. fingolimod more efficacious) than that for DMF twice daily versus placebo in DEFINE/CONFIRM (0.62 vs. 0.71). In adjusted comparisons, the predicted HR for fingolimod versus placebo in the DEFINE/CONFIRM population was lower than in the FREEDOMS population (initial model, 0.51; final model, 0.60). Fingolimod increased median time to disability health states (EDSS 4, 1.0 years; EDSS 6, 1.5 years) and SPMS (1.9 years) compared with DMF. Comparisons with teriflunomide also showed fingolimod increased times to disability health states. **CONCLUSIONS:** Fingolimod is the only oral treatment that has demonstrated a significant effect on 6-month CDP in a clinical trial and our modeling approach suggests that progression to severe disability health states is delayed by fingolimod.

#### PND7

##### EFFICACY OF INHALED ANTIBIOTICS IN CF PATIENTS WITH CHRONIC P. AERUGINOSA INFECTION: A NETWORK META-ANALYSIS

Janssen KI<sup>1</sup>, Higashi K<sup>1</sup>, Jansen J<sup>2</sup>, Doering G<sup>3</sup>, Elborn S<sup>4</sup>, Calado F<sup>5</sup>, Saggkriotis A<sup>5</sup>, Angyalosi G<sup>5</sup>, Balp MM<sup>5</sup>

<sup>1</sup>Mapi, Houten, The Netherlands, <sup>2</sup>Mapi, Boston, MA, USA, <sup>3</sup>Tübingen University, Tübingen, Germany, <sup>4</sup>Queens University Belfast, Belfast, UK, <sup>5</sup>Novartis Pharma AG, Basel, Switzerland

**OBJECTIVES:** To compare the efficacy of tobramycin powder for inhalation (TIP) relative to tobramycin inhalation solution containing 300 mg/5ml of tobramycin (TIS-T) and 300mg/4ml of tobramycin (TIS-B), aztreonam lysine inhalation solution (AZLI), colistimethate sodium solution (colistin) and colistin inhalation powder (colistin-P) for cystic fibrosis (CF) patients with chronic Pseudomonas aeruginosa infection, by updating a systematic literature review (SLR) and network meta-analysis (NMA) with recently published evidence. **METHODS:** The updated SLR was conducted in Medline, Medline in Process, Embase and the Cochrane Library up to 2012. Individual study results were synthesized and indirectly compared with a Bayesian NMA. As some trials included naïve patients (previously not exposed to the treatment) in one arm, and previously exposed patients to the treatment in the other arm, the naïve and exposed arms were considered separate treatment-by-population groups in the network. **RESULTS:** Three new trials were identified and analysed with eleven trials identified in the previous SLR. In naïve populations, TIP is expected to have similar efficacy as TIS-T, TIS-B, and AZLI (difference in % change in FEV1 predicted at week 4 respectively -1.97 (95% Credible Interval -11.84, 8.03), -2.38 (-12.71, 7.98), and 1.53 (-7.04, 10.00)). Compared to colistin-P, TIP is expected to have similar efficacy, however the point estimate is in favour of TIP (11.36 (-1.75, 24.31)). In exposed populations, TIP is expected to have similar efficacy as TIS-T, TIS-B and colistin (respectively -0.78 (-7.65, 5.99), 0.01 (-10.42, 10.46), and -5.75 (-21.23, 9.89)). No data

on naïve and exposed populations was available for colistin and colistin-P, respectively. **CONCLUSIONS:** By conducting an alternative approach where the naïve and exposed arms were considered separate treatment-by-population groups, the full evidence base could be evaluated, including the data for the dry-powder colistimethate sodium inhaler. This will provide relevant information to support clinical decision making in CF.

#### PND8

##### CLINICAL EFFECTIVENESS OF LACOSAMIDE AND ITS IMPACT ON CONCOMITANT ANTIEPILEPTIC DRUGS CONSUMPTION IN THE CZECH REPUBLIC

Klimes J<sup>1</sup>, Vocolka M<sup>1</sup>, Dolezal T<sup>1</sup>, Foitova H<sup>2</sup>

<sup>1</sup>VALUE OUTCOMES, s.r.o., Prague 2, Czech Republic, <sup>2</sup>UCB, s.r.o., Prague 8, Czech Republic

**OBJECTIVES:** To gather data on clinical effectiveness of lacosamide add-on therapy to standard antiepileptic drugs (AEDs) in the Czech Republic. These data were intended as inputs for a cost-effectiveness analysis. **METHODS:** A retrospective multicenter (n=40) data collection of patients with epilepsy treated with lacosamide for 6 months in clinical practice was performed. Information on the number of seizures and concomitant AEDs before and after lacosamide treatment was observed. In this analysis patients reporting at least a 50% reduction in seizure number 3 months after lacosamide treatment were considered responders. Adverse events reported after initiating lacosamide treatment were collected. **RESULTS:** In total, data from 409 patients were collected, 403 had complete data that were analyzed. Mean (SD) age was 40.4 (±14.2) years and the mean (SD) time since diagnosis 18.7 (±12.8) years. In 91% of patients lacosamide treatment was initiated because of resistance to previous AEDs. Most patients suffered from partial (44.2%), secondary generalized (20.1%) or both (34.0%) types of seizures, or other (1.7%). Following lacosamide administration the mean (median) number of seizures decreased from baseline of 40.3 (12.0) to 25.9 (7.0) (per 3 months). Median (mean) number of seizure reduction was equal to 41% (30%), and 45% were responders. Adverse events occurred in 79 pts (19.6%), with somnolence (6.2%) and dizziness (5.5%) most frequently reported. During lacosamide administration, a decrease in concomitant AEDs number was observed. The mean number per patient decreased from 2.2 to 1.7, as did concomitant AEDs used before and after lacosamide administration: levetiracetam (39% vs. 33%), lamotrigine (38% vs. 32%), carbamazepine (34% vs. 29%), valproate (33% vs. 28%) and zonisamide (17% vs. 8%). **CONCLUSIONS:** Results confirm the effectiveness of lacosamide in the reduction of seizure frequency in clinical practice. In addition, findings suggest that adding lacosamide may lead to a reduced use of other concomitant AEDs.

#### PND9

##### COST ANALYSIS OF ANTI-MIGRAINE PRESCRIBING USING A CLAIMS DATABASE TRUSTR

Nelson Mandela Metropolitan University, Port Elizabeth, South Africa

**OBJECTIVES:** To analyse the cost of different anti-migraine products to determine price differences and the impact of generic prescribing. **METHODS:** A retrospective drug utilisation study was conducted on South African medical insurance claims data for 2011. No clinical information was available. **RESULTS:** A total of 797 patients received 1583 anti-migraine products during 2011. The majority of patients (70.14%) were females and 47.05% of patients were between 30 and 49 years old. Only 13.96% of patients claimed their anti-migraine products from the chronic plan of their medical insurance schemes. The average age of patients was 41.61 (SD=14.91) years, with females on average slightly older than males (41.89 years vs. 40.96 years). Clonidine was the most frequently prescribed active ingredient (accounting for 49.21% of the number of prescriptions, yet only for 25.70% of the amount claimed for anti-migraine products). Triptans (selective serotonin (5-HT<sub>1B/1D</sub>)-receptor agonists) accounted for 27.98% of all anti-migraine prescriptions, but accounted for 45.92% of cost. Five different triptans were prescribed. The average cost per sumatriptan prescription was the lowest (R177.64) of all the triptans. Sumatriptan was the only triptan with generic equivalents. Rizatriptan was the most often prescribed triptan, accounting for 18.51% of prescribing frequency and 29.15% of the amount claimed. Tablets and wafers were the preferred dosage forms. Only 0.32% of prescriptions were for triptan nasal sprays and they were on average the most expensive dosage form prescribed (R428.82 per prescription). **CONCLUSIONS:** The results of this study were in agreement with the findings of previous South African studies. The cost saving implications of generic prescribing were clear in this study. Migraine is an expensive condition to treat and is also affecting the economically active sector of the population. Studies investigating the economic impact of migraine will therefore yield valuable results.

#### PND10

##### PRACTICE OF CEREBROPROTECTORS CONSUMPTION IN UKRAINE

Kuznetsov I, Iakovlieva L, Ribka A, Vasileva A, Kyrychenko O

National University of Pharmacy, Kharkiv, Ukraine

**OBJECTIVES:** Despite the lack of evidence-based efficacy of cerebroprotective drugs, one-third of Europe's population uses of these drugs (WHO statistics). The purpose of the study - to research the dynamics of cerebroprotectors consumption in Ukraine. **METHODS:** Determination of cerebroprotectors consumption by means of frequency analysis, ATC/DDD-methodology during 6 months in 2012 in Ukraine. The consumption of cerebroprotective drugs in money term does not give a true picture about the volume of pharmacotherapy using these drugs, that is why ATC/DDD-methodology was used. As a unit of consumption DDD and PDD were used. **RESULTS:** A comparison of outpatient and inpatient cerebroprotective drugs consumption during 6 months in a neurological hospital and in a drugstore in Kharkov, it was found, that 58 trade names (TN) of drugs in the amount of \$ 28218 (the rate of \$ 1: 8,16 UAH on 1.10.12), in the hospital 17 TNs were used in the amount of \$1518. It is shown, that the inpatient consumption of cerebroprotectors is much higher, than outpatient consumption, for example, vincamine - 2,32 DDDs/100 patients and 1,66 DDDs/100 bed-days; piracetam - 2,59 DDDs/100 patients and 0, 15 DDDs/100 bed-

days, respectively. By frequency of outpatient appointments a combined generic drug fezam (234 appointments, prise \$ 5.1), and outpatient cavinton (19 appointments, prise \$ 17.27) were used often. **CONCLUSIONS:** In Ukraine, outpatient cerebroprotective drugs consumption is greater than their consumption in hospital due to the lack of prescriptions control.

#### PND11

##### UTILIZATION OF ANTI SPASTICITY DRUGS IN MULTIPLE SCLEROSIS: ANALYSIS FROM AN ITALIAN ADMINISTRATIVE DATABASE

Mantovani LG<sup>1</sup>, Furneri G<sup>2</sup>, Scalone L<sup>3</sup>, Ciampichini R<sup>2</sup>, Cortesi PA<sup>3</sup>, Fornari C<sup>3</sup>, Madotto F<sup>3</sup>, Chiadini V<sup>3</sup>, Cesana G<sup>3</sup>

<sup>1</sup>Federico II University of Naples, Naples, Italy, <sup>2</sup>Charta Foundation, Milan, Italy, <sup>3</sup>University of Milano - Bicocca, Monza, Italy

**OBJECTIVES:** Spasticity is a common condition among patients with progressive and/or relapsing forms of multiple sclerosis (MS). Current therapies seem to partially control spasticity symptoms, and patients often receive multiple treatments or switch to new treatments to achieve a better control. The objective of this analysis was to assess the current usage of spasticity drugs and relative patterns of utilization among patients with MS, through administrative database analysis. **METHODS:** Using DENALI datawarehouse, we detected MS patients who, during the period January 2000 – December 2009, had at least one disease modifying agent (DMA) prescription. Then the usage of drugs commonly used in spasticity (muscle relaxant drugs, baclofen, tizanidine, clonidine, dantrolene) was evaluated in this cohort of patients, in terms of number of subjects receiving at least one prescription, and number of DDD (defined daily doses) per patient per year. **RESULTS:** From 2000 to 2009, the annual number of patients with MS, receiving DMA treatment raised from 10,746 to 12,594. Concomitantly, the annual number of patients receiving at least one muscle relaxant prescription raised from 5.87% (n=631) to 9.42% (n=1,186). The most prescribed drug was baclofen with few patients receiving other drugs commonly indicated in spasticity (dantrolene, tizanidine and clonidine). A relevant number of patients using muscle relaxants also received other drugs for the central nervous system, although its usage achieved a peak in 2005 (8% of MS patients). The analysis of DDD per patient/year suggested that the usage of muscle relaxant might be almost chronic in these patients (in 2009, 303 DDD per patients per year). **CONCLUSIONS:** Only 10% of patients with MS currently receive active pharmacological treatment, although this condition seems affecting more than 20% of MS patients in Europe. Also, there are not relevant alternatives or second line options to baclofen, which is the most commonly prescribed drug in this condition.

#### PND12

##### USE OF THE FRENCH CLAIMS AND HOSPITALISATIONS DATABASE TO ESTIMATE THE PREVALENCE AND INCIDENCE OF PARKINSON'S DISEASE IN FRANCE

Blin L<sup>1</sup>, Dureau C<sup>1</sup>, Grolleau A<sup>1</sup>, Corbillion E<sup>2</sup>, Jové J<sup>1</sup>, Lassalle R<sup>1</sup>, Poutignat N<sup>2</sup>, Foubert-Samier A<sup>3</sup>, Droz C<sup>1</sup>, Moore N<sup>4</sup>

<sup>1</sup>INSERM CIC-P 0005, Université Bordeaux, Bordeaux, France, <sup>2</sup>Haute Autorité de Santé, Saint Denis La Plaine, France, <sup>3</sup>INSERM U897, ISPED, Université Bordeaux, CHU de Bordeaux, 33076, France, <sup>4</sup>INSERM CIC-P 0005, Université de Bordeaux, CHU de Bordeaux, Bordeaux, France

**OBJECTIVES:** Few studies have assessed the prevalence and incidence of Parkinson's disease (PD) in France. The objectives of this study were to estimate the prevalence and incidence of PD between 2005 and 2010 using a claims and hospitalisations database. **METHODS:** The EGB database is a 1/97 permanent random sample of the French health care insurance system database linked to the national hospital discharge summary database. Data for all adults with full insurance coverage for PD, or hospitalised with main, related, or associated PD diagnosis, or with at least 3 antiparkinson agent reimbursements over a one-year period were extracted for the years 2004 to 2010. A specific and a sensitive PD criterion were defined: i) patients with a medical diagnosis of PD from full insurance coverage or hospitalisation; ii) same patients plus those without a PD medical diagnosis in the database but a drug pattern compatible with this diagnosis (a second set of at least 3 antiparkinson agent reimbursements over another one-year period and no co-medication with extrapyramidal side effects, as well as no antiparkinson agent pattern specific of another indication). EGB estimations were applied to the French population with age and gender standardization to estimate the prevalence and incidence in France. **RESULTS:** Prevalence of PD increased from 0.27% in 2005 to 0.33% in 2010 using the specific definition of disease, and from 0.38% to 0.46% using the sensitive definition. The incidence rate per year was 0.03-0.04% using the specific definition of disease, and 0.05-0.06% using the sensitive definition. Estimated population size was between 180,000 and 255,000 persons in 2010 with approximately 22,000 to 32,000 new patients per year. **CONCLUSIONS:** The prevalence and incidence of PD in France are likely to be within the range of estimations found in the EGB database using the specific and sensitive definitions of disease; results are consistent with that reported internationally.

#### NEUROLOGICAL DISORDERS – Cost Studies

#### PND13

##### BUDGET IMPACT ANALYSIS OF BOTULINUM TOXIN A THERAPY FOR UPPER LIMB SPASTICITY IN THE UNITED KINGDOM

Kurth H<sup>1</sup>, Remak E<sup>2</sup>, Hortobagyl L<sup>3</sup>, Desai K<sup>3</sup>, Abogunrin S<sup>3</sup>, Dinnet J<sup>4</sup>, Gabriel S<sup>4</sup>, Bakheit AM<sup>5</sup>

<sup>1</sup>IPSEN Pharma, Boulogne Billancourt, France, <sup>2</sup>Evidera, Budapest, Hungary, <sup>3</sup>Evidera, London, UK, <sup>4</sup>IPSEN Pharma, Boulogne-Billancourt, France, <sup>5</sup>Moseley Hall Hospital, Birmingham, UK

**OBJECTIVES:** Upper limb spasticity (ULS) secondary to upper motor neurone lesions has a considerable patient and caregiver burden, particularly with regards to pain, activities of daily living and personal care. BotulinumtoxinA (BoNT-A) injections are effective in treating ULS. We developed a budget impact model (BIM) to assess different BoNT-A treatments available in the UK for reducing ULS. We also assessed annual costs of treating each ULS patient with BoNT-A

or best supportive care (BSC). **METHODS:** The BIM was developed from the UK NHS (National Health Service) and Personal and Social Services (PSS) perspective. The status quo scenario assumed the three BoNT-As, Dysport® (abobotulinumtoxinA), Botox® (onabotulinumtoxinA), or Xeomin® (incobotulinumtoxinA), are used in 33%, 52% and 15%, respectively, of patients with ULS receiving BoNT-A in the UK. The new market share scenario assumed an increased proportional use of abobotulinumtoxinA (to 73% in year 5) compared to other interventions. The patients were modelled over a 5-year horizon. Epidemiologic data inputs were from published sources. Unit costs for BoNT-As, other health care costs and non-medical costs came from the British National Formulary and PSS. Resource-use inputs were obtained from UK clinicians. One-way sensitivity analyses for model inputs were conducted. **RESULTS:** Total care costs were decreased by between £425,765 in year 2 and £1,854,601 in year 5 by shifting market share to abobotulinumtoxinA. In the base-case scenario, BSC (no BoNT-A treatment) or with incobotulinumtoxinA or onabotulinumtoxinA cost more per patient per year than abobotulinumtoxinA. Sensitivity analyses showed that number of patients treated with BoNT-As, time-to-re-injection, and dose per injection of abobotulinumtoxinA and onabotulinumtoxinA were the most influential parameters on budget impact, impacting both drug acquisition costs and physician visits. **CONCLUSIONS:** Study findings suggest that increased use of abobotulinumtoxinA compared with incobotulinumtoxinA and onabotulinumtoxinA for ULS in the UK could potentially reduce total treatment costs.

#### PND14

##### THE BUDGET IMPACT OF INTRODUCING BG-12 (DIMETHYL FUMARATE) FOR TREATMENT OF RELAPSE-REMITTING MULTIPLE SCLEROSIS (RRMS) IN CANADA

Dorman E<sup>1</sup>, Kansal AR<sup>1</sup>, Sarda S<sup>2</sup>

<sup>1</sup>Evidera, Bethesda, MD, USA, <sup>2</sup>Biogen Idec, Weston, MA, USA

**OBJECTIVES:** Multiple sclerosis causes significant disability and mortality globally and is especially prevalent in Canada and across Europe. BG-12 is an orally administered disease modifying treatment for relapsing-remitting MS (RRMS) patients currently on the market in the United States and Canada and under review in Europe. A budget impact model (BIM) was developed to assess the financial consequences of introducing BG-12 for the treatment of RRMS in Canada. **METHODS:** A BIM calculated the financial consequences of introducing BG-12 in Canada over three years based on RRMS prevalence, treatment market share, and clinical effects. RRMS prevalence in Canada was derived from published literature and natural relapse rates and disease state distribution from clinical trial data. It was conservatively assumed that 100% of RRMS patients were treated with a disease modifying treatment. BG-12 was assumed to absorb market share proportionally from the following current treatments: interferon beta-1a IM, interferon beta-1a SC, interferon beta-1b, glatiramer acetate, natalizumab, and fingolimod. Treatment efficacy, in terms of reductions in relapse rate, and treatment discontinuation rates were determined from a mixed treatment comparison. Treatment costs (including costs of acquisition, monitoring, and administration) and the cost of relapse were considered. Deterministic one-way sensitivity analyses were conducted to assess the most sensitive input parameters. **RESULTS:** Over three years, the introduction of BG-12 resulted in an average annual increase of CAD279 per treated patient per year, with reductions in costs associated with relapses (CAD192/patient/year) partially offsetting increased drug acquisition costs (CAD471/patient/year). On a population level, the average annual cost increase was CAD16,494,850. The main drivers of budget impact were cost of BG-12, drop-out rates, proportion of RRMS patients treated, and market share assumptions. **CONCLUSIONS:** The acquisition costs of BG-12 for treatment of RRMS are predicted to be partially offset by reduced costs of relapses in the Canadian health care system.

#### PND15

##### BUDGET IMPACT OF EVEROLIMUS FOR TUBEROUS SCLEROSIS COMPLEX (TSC) RELATED ANGIOMYOLIPOMA (AML): UNITED KINGDOM PERSPECTIVE

Whalen JD<sup>1</sup>, Srivastava B<sup>2</sup>, Ozer-Stillman I<sup>1</sup>, Gray L<sup>3</sup>, Price L<sup>3</sup>, Magestro M<sup>2</sup>

<sup>1</sup>Evidera, Lexington, MA, USA, <sup>2</sup>Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA, <sup>3</sup>Novartis Pharmaceuticals UK Limited, Frimley, UK

**OBJECTIVES:** AMLs are benign tumors common in patients with TSC, associated with high morbidity (aneurysm, hemorrhage, chronic kidney disease), and can result in death. Complication risk may correlate with increased AML volume. Historically, AMLs were treated surgically with embolization or tissue-sparing resection. In the EXIST-2 trial, everolimus significantly reduced AML volume in TSC patients. This analysis estimates the cost of reimbursing everolimus for TSC-related AML to the UK health care system. **METHODS:** A Markov model was built to analyze budget impact over five years. The treated population is estimated using TSC and AML prevalence. Adult patients with growing AML  $\geq 3$  cm are assumed eligible for everolimus. Treatment reference costs are from the UK, resource utilization data from The Netherlands, efficacy and safety assumptions from EXIST-2. The model assumed one-year treatment duration. Responding patients ( $\geq 30\%$  AML volume reduction) may restart everolimus upon AML regrowth. Costs are discounted at 3.5% per annum. Sensitivity analyses were conducted. **RESULTS:** Up to 1,474 adults in the UK have TSC-related AML; 30% are assumed eligible for everolimus. On average, 165 patients are estimated to be treated annually with everolimus (Year 1: 88; Year 5: 233) at an average cost of £4,600,000 (Year 1: £2,700,000; Year 5: £6,200,000). Over five years, AML-related medical spending decreased £54,000. Annual per patient treatment cost with everolimus is £31,000. Results were most sensitive to patient prevalence, percent eligible for everolimus, and the percent experiencing  $\geq 30\%$  AML volume reduction. **CONCLUSIONS:** TSC is a relatively rare genetic disease for which everolimus is the only non-surgical treatment that has demonstrated efficacy in reducing AML volume. Reducing AML volume may prevent long-term complications and avoid surgeries, resulting in decreased AML-related medical costs. Further long-term studies are needed to better understand the benefits of everolimus in preventing AML-related morbidity and the associated costs.